Mirum Pharmaceuticals Announces Breakthrough Therapy Designation for Maralixibat for the Treatment of Pruritus Associated with Alagille Syndrome

October 28, 2019

FOSTER CITY, Calif.--(BUSINESS WIRE)--Oct. 28, 2019-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), a biopharmaceutical company focused on the development and commercialization of novel therapies for debilitating liver diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for maralixibat for the treatment of pruritus associated with Alagille syndrome (ALGS) in patients 1 year of age and older.

Breakthrough Therapy Designation is granted by the FDA to investigational medicines intended to treat a serious or life-threatening condition for which preliminary clinical evidence may demonstrate substantial improvement on at least one clinically significant endpoint over available therapy. Breakthrough Therapy Designation is intended to expedite development and review and conveys all of the fast track designation program features, including more intensive FDA interaction and guidance. This program was designed by the FDA to help ensure patients gain access to important new therapies through FDA approval as soon as possible.

The Breakthrough Therapy Designation of maralixibat was granted based on evidence from the ICONIC Phase 2b clinical trial in children with ALGS. Results from the clinical trial were recently presented at the International Liver Congress (EASL).

About Alagille Syndrome

ALGS is a rare genetic disorder in which bile ducts are abnormally narrow, malformed and reduced in number, which leads to bile accumulation in the liver and ultimately progressive liver disease. The estimated incidence of ALGS is one in every 30,000 to 50,000 births in the United States and Europe. In patients with ALGS, multiple organ systems may be affected by the mutation, including the liver, heart, kidneys and central nervous system. The accumulation of bile acids prevents the liver from working properly to eliminate waste from the bloodstream and leads to progressive liver disease that ultimately requires liver transplantation in 15% to 47% of patients. Signs and symptoms arising from liver damage in ALGS may include jaundice, pruritus and xanthomas, which are disfiguring cholesterol deposits under the skin. The pruritus experienced by patients with ALGS is among the most severe in any chronic liver disease and is present in most affected children by the third year of life.

About Maralixibat

Maralixibat is a novel, minimally-absorbed, orally administered investigational drug being evaluated in several rare cholestatic liver diseases for pediatric populations. Maralixibat inhibits the apical sodium dependent bile acid transporter, which results in more bile acids being excreted in the feces, leading to lower levels of bile acids systemically, thereby potentially reducing bile acid mediated liver damage and related effects and complications. More than 1,500 individuals have received maralixibat, including more than 100 children who have received maralixibat as an investigational treatment for Alagille syndrome (ALGS) and progressive familial intrahepatic cholestasis (PFIC). In a Phase 2 PFIC study, a genetically defined subset of BSEP (bile salt export protein) deficient (PFIC2), patients responded to maralixibat, which led to maralixibat receiving Breakthrough Therapy designation from the FDA for PFIC2. In the ICONIC Phase 2b ALGS clinical trial, patients taking maralixibat had significant reductions in bile acids and pruritus compared to placebo. Maralixibat was generally well-tolerated throughout the studies. The most frequent adverse events were diarrhea, abdominal pain and vomiting.

About Mirum Pharmaceuticals

Mirum Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company focused on the development and commercialization of a late-stage pipeline of novel therapies for debilitating liver diseases. The company's lead product candidate, maralixibat, is an investigational oral drug in development for progressive familial intrahepatic cholestasis (PFIC) and Alagille syndrome (ALGS). For more information, visit MirumPharma.com. Follow Mirum on Twitter, Facebook and LinkedIn.

Forward Looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements include statements regarding, among other things, the results, conduct, progress and timing of Mirum's clinical trials and the review and approval process by regulatory authorities of its product candidates. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Words such as "plans," "will," "believes," "anticipates," "expects," "intends," "goal," "potential" and similar expressions are intended to identify forward-looking statements. These forward-looking statements are based upon Mirum's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, risks and uncertainties associated with Mirum's business in general, and the other risks described in Mirum's filings with the Securities and Exchange Commission, including without limitation in its Quarterly Report on Form 10-Q for the quarter ended June 30, 2019. All forward-looking statements contained in this press release speak only as of the date on which they were made. Mirum undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made, except as required by law.

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